

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



WORD OF WELCOME

This year, the Annual Conference of the Inborn Errors Working Party (IEWP) will be held in Zurich, Switzerland, from 25-27 September 2026. It will be a great pleasure to welcome physicians and nurses with a special interest in inborn errors on behalf of IEWP of EBMT.

As usual, the meeting will feature comprehensive sessions on hematopoietic stem cell transplantation and ex-vivo gene therapy across inborn errors of immunity, inborn errors of metabolism, as well as other and new indications.

There will be special scientific sessions and insights from distinguished keynote speakers on immuno-conditioning for HSCT and on Epstein-Barr Virus and autoimmune disease. This year's special session will focus on Chronic Granulomatous Disease from biological mechanisms to novel therapeutic approaches. The Junior's Committee will organise a dedicated session featuring management of complex inborn errors of immunity cases, while the Nurses group will prepare for the first time a dedicated nurse's track with focused presentations.

We are excited to invite you to join us in supporting this key event and we look forward to welcoming you to Zurich!

Maria Ester Bernardo – Working Party Chair

Claire Booth – Working Party Secretary

Tayfun Güngör – Local Organiser

Jana Pachlopnik Schmid – Local Co-Organiser

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



SCIENTIFIC PROGRAMME – MAIN TRACK

Friday, 25 September 2026

13:00-13:45	Registration & Welcome Coffee	
13:45-14:00	Welcome from the organisers Maria Ester Bernardo (IT), Claire Booth (UK), Tayfun Güngör (CH) and Jana Pachlopnik Schmid (CH)	
14:00-16:07	Session 1 Allogeneic HSCT and HSC-GT for IEM Chairs: Giulia Consiglieri (IT) and Robert Wynn (UK)	
14:00-14:07	Case presentation: Disseminated Mycobacterium avium complex infection and Immune Reconstitution Inflammatory Syndrome following Haematopoietic Stem Cell Transplantation for Mucopolysaccharidosis Type II: a complex post-transplant course	Aisling McCann (IE)
14:07-14:27	Biomarkers in ALD, Gene Therapy and Allo-HCT	Troy Lund (US)
14:27-14:47	Long-Term Outcomes of HSC Gene Therapy and Biomarkers in Metachromatic Leukodystrophy	Vera Gallo (IT)
14:47-15:02	Update on the MPS II clinical trial/immune response to transgene and assays	Claire Horgan (UK)
15:02-15:17	Update on the MPS III clinical trial	Claire Horgan (UK)
15:17-15:32	Real-world data for MLD	Valeria Calbi (IT)
15:32-15:47	Platform approach for LSDs with skeletal and neurological involvement: clinical trial design	Maria Ester Bernardo (IT)
15:47-16:07	Mechanisms of immune response after HSC-GT for metabolic diseases	Silvia Gregori (IT)
16:10-16:40	Coffee Break	
16:40-18:50	Session 2 CGD special session from mechanism to novel therapies Chairs: Tayfun Güngör (CH) and Emma Morris (UK)	
16:40-16:47	Case presentation: Chronic Granulomatous Disease: a Cohort of 173 Patients—10-Years Single Center Experience from Egypt	Sohilla Lotfy (EG)
16:47-17:12	EROS and new insights into CGD signaling pathways	David Thomas (UK)
17:12-17:32	CGD - a tough candidate for allo HSCT	Tayfun Güngör (CH)
17:32-17:52	Biologics in chronic granulomatous disease	Jennifer Leiding (US)
17:52-18:07	Gene Therapy in HSC for correcting the oxidative burst in CGD	Giorgia Santilli (UK)
18:07-18:17	The complexities of surviving CGD	Helen Braggins (UK)
18:17-18:27	First inhuman in vivo gene therapy	Emma Morris (UK)
18:27-18:40	Panel Discussion	
18:40-18:50	Tribute to Paul Veys	Persis Amrolia (UK)

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



SCIENTIFIC PROGRAMME – **NURSES TRACK**

Saturday, 26 September 2026

10:30-12:30	Session 1 Care Team Challenges in Patient and Family Care Chairs: Marina Ben Izhak (IL) and Karen Nicholson (UK) Welcome message	
10:30-11:00	Challenges we face every day treating private and international patients	Tanja Stankova (UK)
11:00-11:30	Stakeholder perspectives on Stem Cell Therapy for Patients with Inborn Errors of Immunity (25+5)	Hilda Mekelenkamp (NL)
11:30-12:00	Partnering with Families: Co-Designing information resources for paediatric Gene Therapy	Evey Howley (UK)
12:00-12:30	Psycho-educational intervention for parents after their child was diagnosed with an IEI following a positive NBS test	Patricia Luck (CH)
12:30-13:00	Lunch Break	
13:00-14:22	Session 5 Thymus transplant (Main Track)	
14:22-15:24	Session 6 Advances in GvHD and endothelial complications (Main Track)	
15:25-15:45	Coffee Break	
15:45-16:45	Session 2 Quality of Life and long-term outcomes Chairs: Evey Howley (UK) and Tanja Stankova (UK) Welcome message	
15:45-16:05	Post-GT experience in WAS patients with a focus on quality of life and long-term follow-up	Federico Frascchetta (IT)
16:05-16:25	Developing a pathway for transition from the paediatric to adult long-term follow-up clinic	Karen Nicholson (UK)
16:25-16:45	Back to Life: A Multidisciplinary Clinic for Pediatric Post-BMT Care	Marina Ben Izhak (IL)

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



SCIENTIFIC PROGRAMME – MAIN TRACK

Saturday, 26 September 2026

09:00-09:15	ADA2 updates, including management of patients with dominant negative disease	Isabelle Meyts (BE)
09:15-10:30	Session 3 HSC-Gene therapy for IEIs Chairs: Sung-Yun Pai (US) and Francesca Ferrua (IT)	
09:15-09:25	Overview of current open trials	Claire Booth (UK)
09:25-09:35	Preclinical development of gene therapy for Major Histocompatibility Complex Class II (MHC II) Deficiency	Alessandra Magnani (ES)
09:35-09:40	International XIAP GT consortium	Claire Booth (UK)
09:40-09:50	CD40L moving to the clinic	Daniele Canarutto (IT)
09:50-10:00	P47 CGD LV clinical trial update	Jasmeen Dara (UK)
10:00-10:10	Artemis -SCID	Marina Cavazzana (FR)
10:10-10:20	Etuvetidigene autotemcel approval: current update about rollout/availability, and results from Early Access Scheme	Francesca Ferrua (IT)
10:20-10:30	Initiatives and regulatory innovation to improve access to GT in Europe	Arjan Lankester (NL)
10:30-10:45	Coffee Break	
10:45-11:25	Keynote Lecture Epstein-Barr Virus, autoimmune disease & big data management	Prof. Christian Münz (CH)
11:25-12:10	Session 4 B cells and plasma cell derived treatments Chairs: Carsten Speckmann (DE) and Julian Thalhammer (DE)	
11:25-11:35	Pathology on early B cells defects	Mirjam van der Burg (NL)
11:35-11:45	HSCT experience in XLA	Andrew Gennery (UK)
11:45-11:55	B cell pathology in CVID	Bärbel Keller (DE)
11:55-12:10	Anti-CD19 CAR T cells refractory autoimmune disease	Franco Locatelli (IT)
12:10-13:00	Lunch Break	
13:00-14:22	Session 5 Thymus transplant Chairs: Alexandra Kreins (UK) and Mathias Hauri-Hohl (CH)	
13:00-13:07	Case presentation: Delayed thymic immune reconstitution in paediatric patients with Chronic Granulomatous Disease that underwent allogeneic hematopoietic stem cell transplantation	Laura Marsiglio (CH)
13:07-13:27	Rebuilding Immunity after Paediatric HCT: The Role of Thymic Hormones	Coco de Koning (NL)
13:27-13:47	Sex differences in the human thymus	Viktoria Hennings (SE)
13:47-14:07	Two cases on Challenging referrals for thymic transplants	Tania Masmus (DK) and Zainab Golwala (UK)
14:07-14:22	Discussions on the current state algorithm for decision-making	Alexandra Kreins (UK)

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



SCIENTIFIC PROGRAMME – MAIN TRACK

Saturday, 26 September 2026 (continued)

14:22-15:24	Session 6 Advances in endothelial complications	
	Chairs: Andrew Gennery (UK) and Virginia Meda Spaccamela (CH)	
14:22-14:29	Case presentation: Impact of Pulmonary Disease on Survival and Long-Term Respiratory Outcomes in Children undergoing Haematopoietic Cell Transplantation for Inborn Errors of Immunity	Ho Wai Koo (UK)
14:29-14:39	Light Therapy for oral mucositis: the nurse experience	Karin Schoch (CH) & Svenja Schweizer (CH)
14:39-14:54	Endothelial biomarker dynamics in Sinusoidal Obstruction Syndrome following paediatric allogeneic HSCT	Katrine Kielsen (DK)
14:54-15:09	Paediatric VOD: Still a concern?	Matthias Felber (CH)
15:09-15:24	Pulmonary Vasculopathy after paediatric HSCT - a review of the disease and proposal for a monitoring strategy and early treatment	Antonio Grasso (UK)
15:25-15:45	Coffee Break	
15:45-16:25	Keynote Lecture	
	Immuno-conditioning for SCT and GT	Prof. Markus Manz (CH)
16:25-17:17	Session 7 Precision conditioning	
	Chairs: Thomas Fox (UK) and Arjan Lankester (NL)	
16:25-16:32	Case presentation: Comparison of Cyclophosphamide and Fludarabine in alloreactive settings in vitro	Antonia Reers (DE)
16:32-16:47	Advances in targeted conditioning	Persis Amrolia (UK)
16:47-17:02	PK and model-informed precision dosing of chemotherapy to optimize conditioning	Janel Long-Boyle (US)
17:02-17:17	Alemtuzumab PK/PD to optimize conditioning	Thibaut César (FR)
17:17-18:24	Session 8 Advanced diagnostics and treatment of infections	
	Chairs: Bénédicte Neven (FR) and Jacques Rivière (ES)	
17:17-17:24	Case presentation: Successful Allogeneic HSCT Following Peri-Transplant IFN- γ Blockade With Emapalumab in Complete IFNGR2 Deficiency	Linda Rossini (IT)
17:24-17:44	Fungal infections in CGD: new antifungal drugs, indications for granulocyte transfusions as bridge to HSCT, when to transplant with an active fungal infection	Christa Zerbe (US)
17:44-17:59	When conventional antimicrobials are not enough: new antimicrobial drugs in HSCT, including antibacterials, antivirals (anti-HSV agents) and phage therapy	Natalia Mendoza (ES)
17:59-18:14	Atypical viral infections: Aichi virus	Muriel Schmutz (FR)
18:14-18:24	NGS case report with neurological involvement, case series from Necker's experience with NGS	Cassandre Cremades (FR)

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



SCIENTIFIC PROGRAMME – MAIN TRACK

Sunday, 27 September 2026

09:00-10:30	Session 9 Educational session Management of IEIs in cases with unclear/inconclusive genetic findings Chairs: Lisa Ott de Bruin (NL) and Sujal Ghosh (DE)	
09:00-09:15	Case 1	Filippo Consonni (IT)
09:15-09:30	Case 2	Lisa Ott de Bruin (NL)
09:30-09:45	Case 3	Alexandra Kreins (UK)
09:45-10:05	Transplant decision-making in inborn errors of immunity	Sophie Hambleton (UK)
10:05-10:30	Panel Discussion	
10:30-10:50	Coffee Break	
10:50-11:35	Session 10 IEWP Studies Chairs: Maria Ester Bernardo (IT) and Claire Booth (UK)	
10:50-10:55	NfKB2 and HSCT cohort (update)	Catharina Schütz (DE)
10:55-11:10	P-CID study	Stephan Ehl (DE)
11:10-11:20	FHL2/3 study	Svea Böhm (DE)
11:20-11:30	CHH study	Helja Lång (FI)
11:30-11:35	ADA-SCID study	Maria Pia Cicalese (IT)
11:35-12:50	Session 11 Advances in immune dysregulation Chairs: Tiphaine Arlabosse (CH) and Jana Pachlopnik Schmid (CH)	
11:35-11:42	Case presentation: First haematopoietic cell transplantation as a curative treatment for monogenic SFK-related multisystem vasculitis	Ina Schim van der Loeff (UK)
11:42-11:59	Autophagy/inflammation in WAS	Elizabeth Rivers (UK)
11:59-12:16	STAT6 GOF updates on the current ESID registry study	Julia Körholz (DE)
12:16-12:33	ZNF1 update on current collaborative study	Julius Köppen (CH)
12:33-12:50	NFKBIA clinical spectrum and HSCT strategies	Winnie Ip (UK)
12:50-13:00	Final Remarks Maria Ester Bernardo (IT), Claire Booth (UK), Tayfun Güngör (CH) and Jana Pachlopnik Schmid (CH)	

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



THANKS TO OUR SPONSORS

Silver Partner



Miltenyi Biotec

neovii

Bronze Partner



ensoma



Jazz Pharmaceuticals



IDEOGEN



Orchard
therapeutics
A Kyowa Kirin Company



**Sumitomo
Pharma**

Local Supporter



Biotest

From Nature for Life



MSD